

**Statement of Rep. Henry A. Waxman**  
**National Organization for Rare Disorders (NORD)**  
**30<sup>th</sup> Anniversary of the Orphan Drug Act**

I am delighted to be here tonight to celebrate this tremendous milestone with all of you. And I am honored and touched to receive this recognition.

I am always pleased to have opportunities to talk about the Orphan Drug Act. As the principal author of the Act, I am extremely proud of its many achievements over the past 30 years. In a world where so many people face hopeless odds against getting the medical treatments they need, the Orphan Drug Act has brought genuine hope to the more than 25 million Americans living with rare diseases. It shows what the combined efforts of industry and government can do to bring desperately needed treatments to those who are suffering.

Before its enactment, people suffering from a rare disease had little or no hope that a treatment or cure would be developed, despite the fact that many potential treatments for those diseases existed. Those treatments were simply not being developed because the market for a drug for a given rare disease simply wasn't large enough to give drug companies adequate incentives to bring these potential treatments through the costly drug approval process.

In the early 1980's, when we were looking at the dire situation for people with rare diseases, we frankly weren't sure what to do. We of course had a private pharmaceutical development system in which the government really played no role. So we had to look to the pharmaceutical industry to invest in and bring these orphan treatments to market.

So we had to figure a way to coax the pharmaceutical industry into doing something that was squarely in the public interest. We had to figure out how to make the development of orphan treatments something they wanted to do.

So we created incentives: intellectual-property protections, tax benefits, and regulatory advantages.

In 1983, the Act became law and the rest is history.

I never dreamed the Act would be the success that it's been. I never expected I could say to you today that over 400 orphan drugs have been approved by the FDA—compared to a mere 10 treatments that had been developed by industry prior to the Act—and millions of Americans have benefited from these treatments.

The Orphan Drug Act has been and will remain one of my proudest moments.

Thank you for your hard work and I look forward to continuing our joint efforts to bring hope and treatments to those with rare diseases.